ROYAL PHARMACEUTICAL SOCIETY

NEW MEDICINES, BETTER MEDICINES, BETTER USE OF MEDICINES

Executive Summary: A Guide to the

Science Underpinning Pharmaceutical Practice

May 2014

EXECUTIVE SUMMARY

Medicines are one of the most important aspects of modern healthcare. They can improve quality of life, cure illness and avoid premature death. However, in spite of the ubiquitous nature of medicines, the complexity of the medicines development process is often not fully understood.

This guide, 'New Medicines, Better Medicines, Better Use of Medicines', summarises the important role pharmaceutical science has played and continues to play in the development and use of medicines. It demonstrates the breadth of scientific knowledge and understanding necessary to underpin the full spectrum of pharmaceutical practice.

The guide also highlights the major challenges and opportunities faced when creating new medicines, improving existing medicines or ensuring the better, safer, use of medicines. Finally, it makes a series of seven recommendations for the future.

Pharmaceutical Science

Pharmaceutical science encompasses the basic, applied and social sciences and plays a part in all stages of the journey of a medicine, from its discovery as a new molecule and formulation as a medicine, to its manufacture, approval by the regulatory agencies and ultimate use.

Pharmaceutical science in the UK has a long and excellent record of medicines research and development, being at the forefront of many major advances in modern medicines.

The ever-changing climate of medicines development and use requires a highly educated, multidisciplinary, flexible and well-trained workforce. UK pharmaceutical science has already made huge contributions to improving the health and wealth of the nation, being a net earner for Britain for more than 30 years. It is an important employer and a leading investor, with £4.85 billion[i] spent on UK research and development in 2011. There are challenges ahead for the industry and it is vital to maintain the UK's position to further advance patient health.

New Medicines, Better Medicines

Developing a new medicine is a costly and lengthy process. It is currently estimated that taking a drug from concept to market requires an average of 12 years with costs in the range of £50 million to over £1 billion being widely reported.[ii, iii]

The vast majority of potential drugs never reach market, with most discarded during initial screening. The cost associated with drug failure increases as the drug moves through the development process, with failure in clinical trials frequently costing hundreds of millions of pounds. Both the cost and time taken to bring a medicine to market need to be reduced to ensure the development of new and innovative medicines remains an attractive proposition. New funding mechanisms are needed to incentivise drug development while at the same time ensuring patients receive the best treatments as early as possible.

This need for new incentives is particularly true for antibiotics as the last new class of drug was discovered in 1987. The incentive for pharmaceutical companies to develop new antibiotics is low due to the poor return on investment they provide. This is because they are usually taken for a short period of time, are frequently curative and newer drugs will need to be reserved to treat patients with infections which are resistant to treatment by other antibiotics.

Scientific advances are providing exciting new opportunities for drug development. For example, sequencing of the human genome has allowed the development of medicines for specific groups of patients. This approach, known as stratified (or personalised) medicine, has led to the development of treatments such as Herceptin for patients with breast cancer. Advances in systems biology are improving our understanding of how a patient's genes and lifestyle, environmental factors, and the interaction between them, influences both the disease process and its response to a medicine.

These scientific advances allow medicines to be developed for small numbers of patients based on their particular genetic make-up, extend the life of other drugs previously discarded because of poor efficacy and identify new indications for well-established medicines. Increasingly, tests are being developed to measure biomarkers that identify susceptibility to a disease and a patient's likely response to a medicine. However, such developments are at a relatively early stage, and further research is required if the full potential of these advances is to be exploited.

Many drugs pose significant formulation challenges including how to selectively deliver drugs to their intended site(s) of action in the body, to maximise efficacy and reduce side effects. At present, the vast majority of medicines are given orally. New methods of drug delivery, such as microneedles, (small patches consisting of thousands of small needles that pierce the upper layer of the skin) provide examples of where the pharmaceutical and bioengineering sciences have come together and produced practical and economically viable innovations which should find wide exploitation in the future.

Special formulation issues arise from the complex nature of biologics, for example vaccines, whole cells and body tissues. Their limited stability means cold storage is needed. This is particularly relevant to vaccinations, where the need for cold storage can account for up to 80% of the price and can prohibit their use in many parts of the world.

Another new and exciting treatment area is regenerative medicine where damaged human cells, tissues and even organs are stimulated to repair themselves, thus holding out the promise of a cure for some diseases. One example of this is the use of stem cells to treat failing organs or joints. Further progress in this field will require much research and there are also many ethical issues to be resolved.

While the majority of medicines treat the symptoms of disease, gene therapy offers the possibility of further curative treatments. Although there has been much interest in this field there has only been very limited success and the promise of this form of therapy remains to be realised. To date, the European Medicines Agency has only approved one gene-therapy medicine, Glybera, to treat high levels of lipids in the blood.

Better Use of Medicines

Medicines account for over 12% of the total yearly NHS budget, about £123 billion across GB in 2011/2012. Once marketed, it is important that medicine use is both clinically and cost effective. However, between 30-50% of patients taking medicines for chronic conditions do not take them as directed[iv], leading to avoidable ill health and economic loss to the healthcare system and society in general. Research has shown that 6% of UK hospital admissions are related to adverse drug reactions equating to 4% of hospital bed capacity[v]. Patient non-adherence to medication regimens is a complex problem, although reviewing a patient's medicine(s) and identifying strategies to help them to take their medicine(s) as intended can help improve adherence.

Pharmacists provide evidence-based advice and guidance on medicines to prescribers and patients and contribute to improving health literacy by supplying information that is tailored to an individual patient's needs. Ensuring the best use of medicines, minimising a patient's risk of experiencing adverse events such as side-effects, reducing medication errors and contributing to medicines safety are core activities of all pharmacy services. Some pharmacists are now prescribing medicines for patients, with early indications of benefits to patients with chronic conditions.

The nationwide network of community pharmacies, so crucial for the prescribing, supply and use of medicines, could in the future be a place where non-invasive tests for biomarkers are used to aid the early detection of disease and optimise medicine selection.

Children and older people present particular challenges as they require medicines at age appropriate doses and in acceptable formulations. Furthermore, in older people, a balance must be struck between taking many medicines for several conditions and minimising side effects and unwanted drug interactions. In both patient groups there is often insufficient evidence to make informed medicines choices, a deficiency that must be addressed.

Pharmaceutical science research has also underpinned pharmacy public health and health protection activities and informed policy. Evidence supports the role of community pharmacy in smoking cessation, providing emergency hormonal contraception services and antibiotic stewardship. Further evidence is needed to optimise the sector's contribution to, for example, weight management, alcohol consumption, and cancer detection.

In developing countries, much remains to be done to tackle diseases such as HIV-related disease, malaria, tuberculosis and to control zoonoses. Simple and affordable treatments for diseases endemic in the developing world are urgently required, together with the development of innovative vaccines against these diseases that do not require refrigerated storage. At a global level, the falsified and counterfeit medicines market must be tackled to ensure such medicines do not enter the legitimate medicines supply chain, potentially causing harm to those who take them.

Recommendations

The following seven recommendations arise from the challenges highlighted in the guide. All require collaboration between stakeholders across the scientific community, industry and relevant agencies to realise the ambitions they contain. Only Recommendation 2 deals with a specific group of medicines, namely antimicrobials. This reflects the growing international concerns around the poor stewardship of antimicrobials intended for human and veterinary use and the lack of development of new antimicrobials.

RECOMMENDATION 1 – ENSURING THE SAFE USE OF MEDICINES

- Promote further research into the causes of medication errors in patients and research into interventions to reduce those errors
- Ensure consideration is given to the safe use of medicines at all stages, from the discovery of a drug to its administration to a patient as a medicine, and that patients understand the risks and benefits of their medication
- Improve pharmacovigilance and reporting of suspected adverse drug reactions by healthcare professionals and patients to identify any safety issues following launch of a medicine
- Encourage developments in toxicology testing, predictive pharmacokinetics, drug delivery, clinical trial design and age related formulations to aid development of safer medicines.

RECOMMENDATION 2 – STIMULATING NEW ANTIMICROBIAL DEVELOPMENT AND IMPROVING ANTIMICROBIAL STEWARDSHIP

- Educate the public and patients on the use of antimicrobials and their place in therapy
- Encourage further development of antimicrobial stewardship by healthcare professionals to maintain the effectiveness of current and any future antimicrobials
- Support the discovery and development of new antimicrobials or treatment methods, by developing new financial incentives.

RECOMMENDATION 3 – ADOPTING NEW TECHNOLOGIES

- Educate the public and patients about the ethical and moral issues surrounding the use of new technologies and medicines such as gene therapy, regenerative medicine, therapeutic vaccines and stratified medicine
- Ensure new technologies and medicines fulfil their potential
- Encourage the development of appropriate models of reimbursement to support the use and development of new technologies.

RECOMMENDATION 4 – SUPPORTING THE DEVELOPMENT OF NEW AND INNOVATIVE MEDICINES

- Encourage the adoption of new technologies and innovative approaches that assist in drug target identification, reduce drug attrition, optimise medicines development and clinical trials and improve the safety profile of medicines
- Facilitate the supply of new and innovative medicines, and reduce the cost and time to bring these medicines to the patient
- Streamline and reduce the regulatory burden associated with approval, particularly of new and innovative medicines, while continuing to ensure patient safety
- Encourage participation and transparency in clinical trials.

RECOMMENDATION 5 – INCREASING THE EVIDENCE BASE FOR PHARMACY

- Increase the health services research expertise within the profession
- Demonstrate the clinical and cost effectiveness of NHS pharmacy services by means of well-conducted, definitive trials that are appropriately funded to enhance the role of pharmacy in the treatment of patients.

RECOMMENDATION 6 – SUPPORTING PHARMACEUTICAL SCIENCE IN THE UK

- Encourage investment in scientific education and training to ensure a highly skilled and adaptive pharmaceutical science workforce
- Ensure that the UK remains a major player in the development of new and innovative medicines by expanding current Government initiatives aimed at making the UK an attractive location for companies of all sizes
- Increase support for more academic/NHS/industrial partnerships.

RECOMMENDATION 7 – IMPROVING ACCESS TO MEDICINES AT A GLOBAL LEVEL

- Tackle disease in developing countries and ensure the equitable access of quality medicines to all patients
- Support the responsible re-use of medicines and improve access to medicines in developing world communities thereby improving health
- Prevent harm to patients by the removal of falsified and counterfeit medicines from the legitimate medicines supply chain and illegal supply through the internet.

References

[i]The Association of the British Pharmaceutical Industry (http://www.abpi.org.uk/industry-info/knowledge-hub/randd/Pages/expenditure.aspx) accessed 2 April 2014

[ii]DiMasi, J.A. and Faden, L.B. Competitiveness in follow-on drug R&D: a race or limitation. Nature Reviews Drug Discovery (2011) **10** 23-27.

[iii]DiMasi, J.A. et al. Trends in risks associated with new drug development: success rates for investigational drugs. Clinical Pharmacology and Therapeutics (2010) **87** 272-277.

[iv]Padwal, R.S. et al. Long-term pharmacotherapy for obesity and the overweight. Cochrane Database of Systematic Reviews (2003), Issue 4. Art. No.: CD004094. DOI: 10.1002/14651858.CD004094.pub2. (http://onlinelibrary.wiley.com/doi/10.1002/14651858.CD004094.pub2/pdf/standard – accessed 9 October 2013).

[v]Pirmohamed, M. et al. Adverse drug reactions as cause of admission to hospital: prospective analysis of 18,820 patients. British Medical Journal (2004) **329** 15-19.

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The Royal Pharmaceutical Society is the professional body for pharmacy, pharmacists and pharmaceutical scientists in Great Britain. The Royal Pharmaceutical Society leads and supports the development of the pharmacy profession including the advancement of science, practice, education and knowledge in pharmacy, as well as promoting public understanding of pharmacy so that its contribution to the health of the nation is understood and recognised. In addition, the Royal Pharmaceutical Society promotes the profession's policies and views to a wide range of external stakeholders in a number of different forums.

New Medicines, Better Medicines, Better Use of Medicines – A Guide to the Science Underpinning Pharmaceutical Practice represents the views of the Society's Pharmaceutical Science Expert Advisory Panel. The Panel is an independent advisory panel of the Royal Pharmaceutical Society composed of 17 leading figures in pharmaceutical science from academic, industrial, regulatory, hospital and community practice from across Great Britain whose remit is to provide strategic direction and assess future developments in pharmaceutical science to the Royal Pharmaceutical Society on critical issues facing pharmacy that impact on patients and the public.

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